hypothyroidism and hypogonadism. Little is known regarding these late effects after newer reduced intensity conditioning (RIC) regimens without irradiation. Our study goal was to evaluate late endocrine effects after RIC HSCT in pediatric and young adult patients.

Methods: An IRB approved retrospective chart review was performed of 120 children and young adults at our center, who received a single RIC without radiation for HSCT between 2004 and 2012 and survived at least 1 year. Analysis was grouped by age (<2 years and ≥2 years), and diagnosis (HLH/XLP, other immune disorders (PID), and metabolic or genetic disorders). Steroid therapy was defined as glucocorticoids administered either before HSCT or beyond 2 months following HSCT. Height for age z-score (HAZ) and BMI z-scores (BMI-Z) were examined separately using linear mixed effects models.

Results: Subjects age 2-17y with height (n=103) and weight (n=120) data both prior to and at least 1 year following HSCT were analysed for growth. The mean follow-up was 3.2 years. All groups displayed short stature before (HAZ = -1.33) and after HSCT (HAZ = -1.35) (p=0.66). After HSCT, younger children with HLH/XLP grew better (HAZ =-3.36 vs -1.35, p= 0.002), while older subjects had worsening (HAZ =-.61 vs -.99, p= 0.004), although all remained short. Overall, subjects receiving steroid therapy were shorter than untreated patients (p=0.02). After HSCT, older subjects with HLH/XLP became thinner (BMI-Z= 1.29 vs. 0.61, p=0.003), and similarly in metabolic or genetic disorders (BMI-Z= 0.56 vs. -0.77, p<0.001). There was a trend toward increased BMI-Z among younger children in these same groups. Thyroid function testing was performed on 77 subjects following HSCT. Eleven (14%) had evidence of primary hypothyroidism, 5 (7%) had central hypothyroidism, and 2 (3%) had evidence of primary hyperthyroidism. Of the 66 subjects with 25-OH vitamin D levels, 46 (70%) were low (<30 ng/mL). Bone densitometry by DXA scan was below -1 SD in 16 of 21 evaluable subjects with an average Z-score of -1.8 SD (0.7 to -4.9 SD) at median duration after HSCT of 2.2 years.

Conclusions: Despite using RIC, children and young adults still have significant late endocrine effects following transplant. Algorithms for early detection of endocrine late effects should be implemented for RIC transplant survivors. Further research is required in order to compare post-transplant endocrine effects after RIC to those after standard chemotherapy protocols.

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Renal Complications in Fanconi Anemia (FA) Patients Undergoing Hematopoietic Cell Transplant (HCT) Using a Uniform Radiation-Free Approach

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Background: Renal and urological congenital abnormalities in patients with FA raise concern for renal dysfunction and may affect their eligibility to undergo HSCT or their post-transplant course and outcome. Some of these patients (or their physicians) may opt not to proceed with HSCT due to concerns of adverse outcome. We describe renal anomalies and associated dysfunction, and their impact on outcomes in a cohort of patients with FA undergoing HSCT.

Methods: A retrospective chart review of 27 patients with FA who underwent HSCT between March 2010 and May 2014.

Table 1
Patient characteristics

Characteristics	Number or Median(range)
Number of patients	27
Pre-HSCT nuclear GFR (mL/min/1.73m2)	
• Patients with renal abnormalities (n=12)	97 (55-131)
Mild Renal impairment (GFR<90)	4
Moderate Renal impairment (GFR<60)	2
• Patients without renal abnormalities (n=15)	124 (71-199)
Mild Renal impairment (GFR<90)	2
Post-HSCT renal complications	
• Acute kidney injury (doubling of creatinine)	12
• Mild Renal impairment (GFR<90mL/min)	14
• Moderate Renal impairment (GFR<60mL/min)	10
• Severe Renal impairment requiring temporary RRT	1
Attributable Causes for renal complications (not mutually exclusive)	
• Transplant associated thrombotic microangiopathy	9
• BK hemorrhagic cystitis	11
• Nephrotoxic medications	6
• Hepato-renal syndrome due to VOD	1
Early withdrawal of CSA due to renal impairment	11
Post-HSCT Renal Function	
• Recovery to GFR>60mL/min at least	
In patients with mild renal impairment post-HSCT (GFR<90mL/min)	13/14
In patients with moderate renal impairment post-HSCT (GFR<60mL/min)	5/10
• Chronic Kidney Disease without dialysis (GFR 35-40mL/min)	2

Results: 12 patients had structural renal/urological abnormalities at baseline. Median nuclear GFR for these patients was 97 mL/min/1.73sq.m (range: 55-131) compared to 124 mL/min/1.73sq.m (range: 71-199) in those without renal anomalies (p=0.03). Additional patient characteristics and results are shown in Table 1.

Post-HCT renal complications included acute kidney injury and decreased renal function measured by Cystatin C GFR. One patient required renal replacement therapy. Medication doses were adjusted based on Cyst C GFR for all patients.

The majority of patients (13/14) with renal impairment pre-transplant recovered to GFR>60mL/min post-transplant. 2 patients with anomalies and pre-HCT GFR <60 mL/min/1.73sq.m are long term survivors, albeit with modest chronic kidney disease (CKD) but without the need for dialysis.

Conclusion: Patients with structural renal abnormalities had lower but surprisingly clinically acceptable pre-HCT GFR. Two patients with anomalies and pre-HCT GFR <60, are long term survivors, showing that patients with renal anomalies/marginal renal function can consider the option of HCT. Routine weekly Cystatin C-GFR allows careful and individualized medication dosing leading to improved outcomes.

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Improved Outcomes in Patients with Dyskeratosis Congenita (DC) Undergoing Allogeneic Hematopoietic Cell Transplantation (HCT) Using Reduced Intensity Conditioning

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Table 1
Patient demographics

Characteristic	Number/Median (range)
Number of patients	7
• Male	4
• Female	3
Age at diagnosis (years)	6.75 (1.3-12.5)
Telomere length <1^{st} centile	7
Genetic Mutation	
• TINF	2
• DKC1	1
• TERT	2
• RTEL1	1
• Unknown	1
Indication for HSCT	
• Bone Marrow Failure	6
• MDS	1
Pre-$HSCT_s$ (in 4 patients)	
• Mild decreased DLCO	1
• Mild restrictive disease	1

Table 2
Transplant characteristics

Characteristic	Number/Median (range)
Age in years at HCT	7.12 (1.7-13.2)
Donor Source	
• MSD	1
• URD	6
Donor HLA matching	
• Full match	6
• One antigen mismatch	1
Cell dose - Median TNC ($\times 10^8$/kg)	8.8 (5.5-10)
Neutrophil engraftment (days)	12 (10-16)
Platelet engraftment (days)	20 (14-35)
Mixed chimerism	0
GVHD	
• Acute GVHD	2
-Skin Grade II	1
-Gut Grade III	1
• Chronic GVHD (limited)	1
Viral infection	
• CMV	4
• EBV	3
• BK	3
• Adenovirus	1
Overall outcome	
• Alive	5
• Dead	2
Cause of death (post HCT day of death)	
• IPS	154
• MRSA sepsis	858

TNC–Total Nucleated Cells, IPS–Idiopathic Pneumonia Syndrome, MRSA-Methicillin-resistant Staphylococcus aureus.

Background: HCT is the only curative option for progressive marrow failure and/or myelosdysplastic syndrome or leukemia associated with DC. HCT for DC is limited by a high incidence of treatment-related mortality; related to underlying chromosomal instability resulting in sensitivity to chemotherapy and radiation. We report our experience using a reduced intensity conditioning (RIC) regimen without radiation for HCT in patients with DC.
Methods: Retrospective chart review was performed with IRB approval.
Results: 7 patients with DC underwent HSCT using a RIC regimen consisting of alemtuzumab, fludarabine, and lower dose melphalan between September 2010 and April 2014. Patient demographics and disease characteristics are shown in Table 1.

1 patient with MDS and DC did not receive alemtuzumab to avoid mixed chimerism. The remaining 6 patients all received alemtuzumab at age appropriate dosing (on Day -22 to -18, or day -14 to 10 or day -6 to -2). All patients received fludarabine from days -8 to -4 (30-40mg/m^2/dose). Melphalan dose was reduced by 50% to 70mg/m^2/dose, to avoid excessive toxicity related to baseline chemo-sensitivity. GVHD prophylaxis consisted of cyclosporine and steroids or MMF. None of the patients had pre-existing hepatic dysfunction.

All patients engrafted and none developed mixed chimerism. See Table 2 for additional results. 2 patients developed acute GVHD, one with grade 2 skin GVHD that evolved in to limited chronic skin GVHD and the second patient developed grade 3 gut GVHD in the setting of pre-existing colitis.

5 patients remain alive and well (100% engrafted) at a median follow up of 28 months (range 5-47.6).
Conclusion: A RIC regimen containing alemtuzumab, fludarabine, and lower dose melphalan (without radiation) results in durable engraftment rates, acceptable toxicity and improved overall survival in patients with DC undergoing HCT.

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Reduced Incidence of Infections in Very Young Children Undergoing Allogeneic Hematopoietic Stem Cell Transplantation

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Background: Opportunistic infections significantly contribute to morbidity and mortality after HSCT. We hypothesized that there is a difference in the infection rate based on age at HSCT.
Methods: We retrospectively examined the medical records of 307 pediatric allogeneic HSCT recipients between 2008 and 2012 at our institution to include all positive infectious results (bacterial, viral and fungal). Screening practices were consistent during the time period.

Table 1
Total number of infections per age group

Age (years)	0-2	2-5	>5	
	n=78	n=58	n=170	p-value
GPC	16	18	44	0.38
GNR	21	7	31	0.09
Mold	1	0	5	0.54
Yeast	3	4	11	0.72
Viral	42	43	126	0.006
Adeno	12	21	37	0.02
BK	2	6	52	<0.0001
CMV	10	13	42	0.1
EBV	20	19	53	0.61
HHV6	4	7	11	0.25
Respiratory	16	9	41	0.38
Flu	4	0	6	0.27
Metapneumo	4	2	6	0.86
RSV	5	5	18	0.59
Paraflu	2	4	10	0.47
Rhino	6	4	11	0.95
Noro	7	5	13	0.88
Parvo	0	0	7	0.07
Rota	2	0	3	0.59
Other	0	0	1	1

GPC- gram positive cocci, GNR- gram negative rod, UTI- urinary tract infection, CMV- cytomegalovirus, EBV- Epstein-Barr virus, HHV6- human herpes virus 6, Flu- Influenza A or B, RSV- respiratory syncytial virus